Rare Diseases Europe

EURORDIS
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Jasmine, UK, Late infantile Batten disease

1st European Rare Disease Day

29 February 2008

“A rare day
for very special people”

Welcome Pack for National Alliances

www.eurordis.org

Content

Laura, Finland, Dysmelia

1. How to use this welcome pack

Efraim & Ester, Portugal, Sickle Cell Disease

- **Read the whole pack.** It will give you background information on the European Rare Disease day and will help you understand the choices that were made and how they will help us achieve our main goal of awareness raising.

- **Spend time thinking about the theme that was chosen for the day.** If necessary, according to the rare disease environment in your region or country, think about a sub-theme or subtitle that would translate the general theme to your own conditions. These conditions vary a lot from country to country, and so might the sub-theme. (section 5)

- **Translate the motto in your own language.** (section 5)

- **Decide on the specific targets** of the Rare Disease Day for your country or region. (section 9)

- **Decide on the specific objectives** of the Rare Disease Day for your region or country; for example, you may not want to set up fund-raising objectives for 2008. (section 10)

- **Read the suggestions for local events and activities** and think about which ones you could organise in your region or country. (section 12)

- **Complete, personalise and translate the press release** to be sent to the media in your region or country. (section 13)

- **Download logos and posters to be used for the Rare Disease Day.** (section 13)

- **You can use the whole pack or only part of it for your activities.** You may need to translate it in your own language to share it with your members.

- **For any questions, please contact Anja Helm.** (anja.helm@eurordis.org)

2. Why a European Rare Disease Day?

Jonathan, Germany,
Maroteaux-Lamy syndrome

- **Because we constantly need to raise awareness on rare diseases** among decisions makers, health professionals and the general public. Information is key to improving living conditions for rare disease patients; raising awareness is therefore one of our primary goals
- **Because acting simultaneously in Europe can ensure the voice of rare disease patients is heard by more people**
- **Because rare diseases are a public health priority** today in the European Union
- **Because a day focussed on rare diseases can bring hope and information** to people living with rare diseases, their carers and their families
- **Because Europe is the focus of most of our actions today**; we hope that in the future this European Rare Disease Day can become a World Rare Disease Day. Focussing on Europe is a first step
- **Because we want equity in access to care and treatment** for rare disease patients in Europe
- **Because we need an action that can bring all stakeholders of the rare disease community together** with the same goal
- **Because we need more funds** for research and care, and **more research** and efforts directed towards rare diseases
- **Because we need to keep fighting** for rare disease patients…
- **Because we need to coordinate policy actions at national level** in the different European countries

3. Why the 29th of February?

Jean, France, Friedreich ataxia

- **Because the 29th of February is a rare day:** It only happens every 4 years and therefore illustrates the concept of rarity
- The other years, the Rare Disease Day will take place on the last day of February: **28th of February**
- **Because there are less days for international causes organised at the beginning of the year.** Most of them happen towards the end of the year, between October and December. Having the Rare Disease Day stand out should serve our cause better

4. Why start in 2008?

Jacob, UK, Spinal muscular atrophy

- **Because the European Commission in currently preparing a Communication on Rare Diseases and a Council Recommendation on Rare Diseases.** It is anticipated that the Communication be adopted by the European Parliament in 2008. The Communication and the Recommendation are the most important events for rare diseases in Europe since the 1999 EU Regulation on Orphan Medicinal Products. *The Communication is the tool that will allow us to further promote rare diseases as a public health priority in EU and national public policies.*

- **Because there's never been a time with so many synergies between the European and the national level** (Commission Communication, national plans for rare diseases, European reference networks of centres of expertise).

- **Because we have succeeded in making rare diseases a public health priority in the European Union,** but we need to fight so that they remain so. For the period 2008-2013 a new Public Health Programme should replace the existing one (if approved by the Council and the Parliament): we need to make sure that rare diseases are still a priority of the new Programme.

- **Because National Alliances are ready to collaborate on a large-scale event for patients living with a rare disease.**

5. The theme for 2008

Arman, Armenia, Phenylketonuria,

- **The motto for the 2008 European Rare Disease Day is:**

  "A rare day for very special people"

- **The theme for the 2008 European Rare Disease Day is**

  "Rare Diseases: a Public Health priority"

- **This year's theme was chosen for its broadness**. 2008 is the first year we will be celebrating a Rare Disease Day in Europe. Some of our main targets, the general public in particular, are not yet fully aware of what rare diseases are, what impact they have on patients' lives, and what needs to be done. We therefore needed to choose a theme that was broad enough to allow us to **introduce the concept of rare diseases as a public health priority.**

  This concept needs to be looked at in the **current context of development of synergies between the European level and the national level.** Three "hot" topics illustrate these current synergies:

  - **the new Commission Communication on Rare Diseases,**
  - **the development of national plans for rare diseases, and**
  - **the development of European reference networks of centres of expertise**

This year's theme encompasses those three issues, which must be adapted to the local and national environments (for example, some countries have a national plan in place, others are in the process of creating one, and others don't have anything yet).

- **For background information on:**
    - ❖ ***Rare Diseases as a Public Health priority***: see section 6 of this welcome pack
    - ❖ ***The new Commission Communication***: see section 7 of this welcome pack
    - ❖ ***National plans for rare diseases***:
        - English:
            - o http://www.eurordis.org/article.php3?id_article=954
            - o http://www.eurordis.org/IMG/pdf/EN_french_rare_disease_plan.pdf
        - French
            - o http://www.eurordis.org/IMG/pdf/EN_french_rare_disease_plan.pdf
            - o http://www.eurordis.org/article.php3?id_article=958
        - German
            - o http://www.eurordis.org/IMG/pdf/DE_franzosischer_nationalplan_seltene_erkrankungen.pdf
            - o http://www.eurordis.org/article.php3?id_article=974
        - Italian
            - o http://www.eurordis.org/article.php3?id_article=966
            - o http://www.eurordis.org/IMG/pdf/IT_piano_nazionale_francese_malattie_rare.pdf
        - Portuguese
            - o http://www.eurordis.org/article.php3?id_article=970
        - Spanish
            - o http://www.eurordis.org/article.php3?id_article=962

  - http://www.eurordis.org/IMG/pdf/SN_plan_nacional_frances__enfermedades_raras.pdf

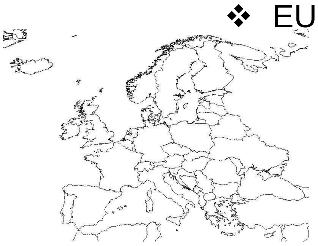
- ***European reference networks of centres of expertise***:

  - English: http://www.eurordis.org/article.php3?id_article=1533
  - French: http://www.eurordis.org/article.php3?id_article=1537
  - German: http://www.eurordis.org/article.php3?id_article=1553
  - Italian: http://www.eurordis.org/article.php3?id_article=1545
  - Portuguese: http://www.eurordis.org/article.php3?id_article=1549
  - Spanish: http://www.eurordis.org/article.php3?id_article=1541

6. Rare Diseases: a Public Health priority

Yuliya, Ukraine, Spinal muscular atrophy

- **The rare disease patient is the orphan of health systems, often without diagnosis, without treatment, without research, therefore without reason to hope.**
- **Rare diseases are life-threatening or chronically debilitating diseases** with a low prevalence and a high level of complexity. 6000 to 8000 rare diseases have been identified, affecting 25 million European citizens. Patients with very rare diseases and their families are particularly isolated and vulnerable. The life expectancy of rare disease patients is significantly reduced and many have disabilities that become a source of discrimination and reduce or destroy educational, professional or social opportunities.
- **Research on rare diseases is scarce**
- **The lack of specific health policies and the scarcity of expertise** translate into delayed appropriate diagnosis and difficulty of access to care. The national healthcare services for diagnosis, treatment and care of rare disease patients differ significantly in terms of their availability and quality. European citizens have unequal access to healthcare services and to orphan drugs.
- **Since 1999, the European Union has taken measures to fight against rare diseases** and their impact on patients’ lives, and has made **rare diseases a priority of its public health programmes:**
  - ❖ EU Regulation on Orphan Medicinal Products (1999)
  - ❖ EU Regulation on Paediatric Drugs (2006)
  - ❖ EU Regulation on Advanced Therapies (2007)
  - ❖ Community Action Programme in the Field of Public Health (1999-2003)
  - ❖ Community Action Programme in the Field of Public Health (2007-2013)
  - ❖ EU 7th Framework Programme for Research (2007-2013)



- Over the last few years, a certain number of Member states have developed **National Plans for Rare Diseases**

- **For background information, see** the “Princeps paper” developed by Eurordis and its members:
    - English:
        - http://www.eurordis.org/IMG/pdf/Princeps_document-EN.pdf
        - http://www.eurordis.org/article.php3?id_article=918
    - French
        - http://www.eurordis.org/IMG/pdf/Princeps_document-FR.pdf
        - http://www.eurordis.org/article.php3?id_article=922
    - German
        - http://www.eurordis.org/IMG/pdf/Princeps_document-GM.pdf
        - http://www.eurordis.org/article.php3?id_article=988
    - Italian
        - http://www.eurordis.org/IMG/pdf/Princeps_document-IT.pdf
        - http://www.eurordis.org/article.php3?id_article=930
    - Portuguese
        - http://www.eurordis.org/article.php3?id_article=934
    - Spanish
        - http://www.eurordis.org/IMG/pdf/Princeps_document-SN.pdf
        - http://www.eurordis.org/article.php3?id_article=987

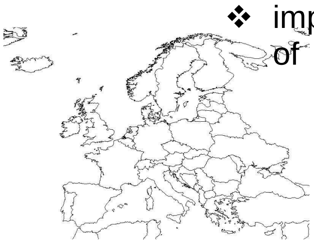
7. The Commission Communication on Rare Diseases

Beau, Belgium, Cystic fibrosis

- **Regulations and Directives, the most common types of acts used by the EU,** are legally binding for Member States and used by the EU to implement its powers. In certain areas such as Public Health, which are the Member States' responsibility, the EU cannot issue binding acts. As a consequence, to help Member States formulate and implement coordinated objectives and strategies in those areas, the EU issues non-legally binding acts called ***Recommendations***. To introduce a new act such as a Recommendation – or to explain its planned actions in a certain domain – the Commission often uses what is called a ***Communication****.* Even if not legally binding for Member States, Recommendations and Communications carry much political weight.

- **The new Communication on Rare Diseases (and hopefully the Council Recommendation that will ensue) will shape the future strategy of Community action in the field of rare diseases for public health, research, and therapy development.** In other words, it is the best tool to further promote rare diseases as a public health priority. The Communication will play a crucial role at the national level, by facilitating the definition of priorities and guidelines for the creation of National Plans on Rare Diseases. It will feed the ongoing reflection on European Reference Networks of Centres of Expertise and will impact national and EU strategies for rare diseases. Patient associations will be able to use the outcomes of this process for their advocacy activities at the national level.

- **The objectives of the Communication are to strengthen the cooperation between the various EU programmes, to encourage Member states in developing national health policies, and to ensure that common policy guidelines are shared everywhere in Europe.** This will be achieved by:
    - improving knowledge and identification of rare diseases (Common EU definition, better codification and classification, and establishment of a list of rare diseases)
    - improving diagnosis and care of patients with rare diseases (Dissemination of appropriate information, development of national/regional centres of



expertise and EU reference networks of centres of expertise, development of e-Health in the field of rare diseases, availability and accessibility of accurate clinical tests, evaluation of population screening strategies for rare diseases, equal access to orphan drugs, specialised social services, coordinated compassionate use programme etc)

  - accelerating research and developments in the field of rare diseases and orphan drugs (Databases, registries, repositories and biobanks, coordination between Member States' funding agencies, and intensification of therapeutic research)
  - empowering patients with rare diseases at individual and collective level
  - coordinating policies and initiatives at Member State and EU level (Adoption of national plans for rare diseases, development of health indicators, European conferences on rare diseases, establishment of a Community Agency for rare diseases)

- **A first draft of the Commission is to be issued by mid-September 2007.** This draft will be sent for comments to the members of the Rare Disease Task Force, the EU High Level Group on Health Services and Medical Care, the COMP, and Eurordis. A second draft will be finalised in October. The official public consultation will be launched early November 2007. The Communication will be presented at ECRD 2007 Lisbon. It is anticipated that the EU Parliament and the Council will discuss the Communication under Slovenian Presidency (First semester 2008), and that it could be approved under French Presidency (Second semester 2008).

8. Organisation principles

Michaël, France,
Mitochondrial Cytopathy

- **Eurordis and National Alliances are the organisers of the day**
    - *Role of Eurordis:*
        - Funding of European-level events (in Brussels)
        - European coordination
        - Decision on dates, themes and content
        - Work with graphic designer for European identity (logo, visuals)
        - Development of some of the tools
        - Evaluation of results and analysis at European level
        - Rare Disease Day Website
    - *Role of National Alliances:*
        - Local actions
        - Adaptation of common tools and development of own tools
        - Funding for local actions
        - Data gathering (results) & evaluation
        - Press / media contacts

- **The European Rare Disease Day is an annual, awareness-raising event**

- **The Rare Disease Day remains flexible at the local level**

- *Duration:* depending on their individual and national environment, National Alliances can choose to organise the Rare Disease Day around one single day, one weekend, several days, or a week

- *Regularity:* it is preferable for the day to happen every year in a given country; however, a National Alliance could decide differently, if there are reasons for it

- *Fund-raising:* some National Alliances may prefer not to make the day a fund-raising event

- *Tools used*: Eurordis suggests a variety of tools for National Alliances, which they are free to adapt and translate if necessary: Alliances can use of their own

- *Targets*: they may vary per country

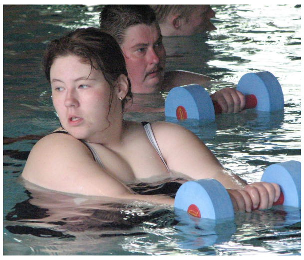
9. Who is the Rare Disease Day for?

Letitia, Germany, Epidermolysis bullosa dystrophica

- **The primary targets of the European Rare Disease Day are** (in decreasing order):
    - ❖ General public
    - ❖ European and national authorities and policy makers
    - ❖ Healthcare professionals
    - ❖ Researchers
    - ❖ Media (as a tool to reach the general public)

- **Secondary targets could be:**
    - ❖ Patient associations and patients, including those without an official organisation to represent them because of the rarity of their disease
    - ❖ Pharmaceutical industry

10. What are the objectives of the day?



Marianna, Finland, Prader-Willi Syndrome

- **Raise awareness of rare diseases** and of their impact on patients' lives, **and reinforce their importance as a public health priority**
    - ❖ 6000 – 8000 rare diseases
    - ❖ 25 million rare disease patients in the European Union
    - ❖ No existing, effective cure
    - ❖ 75% of rare diseases affect children
    - ❖ Lack of access to correct diagnosis, of quality information, of scientific knowledge on rare diseases
    - ❖ Heavy social consequences for patients; lack of appropriate quality healthcare; inequities and difficulties in access to treatment and care
    - ❖ Importance and role of the European Union in creating and implementing a sustainable framework for the prevention and treatment of rare diseases for ALL citizens
- **Provide hope, information and help to patients affected by chronic, rare diseases,** in particular those for whom a support network is not available or known
- **Reinforce the European collaboration in the fight against the impact of rare diseases on the lives of patients and their families**
- **Be the starting point for a more inclusive, worldwide Rare Disease Day**
- **Raise funds to pursue our action,** at both European and national levels

11. What will happen at the European level?

Francesca, Diastrophic dwarfism

- **Eurordis is organising a Public Hearing at the European Parliament, in Brussels.** This event targets European policy makers and the media. Its objectives are to raise awareness on rare diseases and to stress the importance of the role of the Commission and of the EU in fighting against rare diseases in Europe.

- **The hearing will take place on Tuesday March 4th 2008, from 14.30** to 17.00 and is hosted by MEP Frédérique Ries.
  - *Theme:* European Rare Disease Day and future Commission Communication on Rare Diseases
  - *Commission's Sponsor:* Mrs Frédérique Ries, MP
  - *Commission's representative:* Commissioner for Health, Mr Kyprianou or a high-level representative of DG Sanco
  - Other contacts at the Commission, including the Rapporteur for the Commission Communication on Rare Diseases
  - *Eurordis representatives:* Terkel Andersen (President)
  - Patients and patient representatives, patient group representatives
  - Selected media
  - *An information kit* will be developed by Eurordis for the people present at the function (Rare Diseases as a public health priority, Commission Communication, list of activities per country, and a press release)

- **A press release will be issued by Eurordis**

12. What will happen in the different countries?

Francesco, Italy, Williams syndrome

- **The various activities taking place around the Rare Disease Day in each country are up to the National Alliance(s) active in the country.** Hereafter you will find a list of possible activities for your country, according to your resources, time, strategy, and funds available
- **You will need to provide a list of those activities well ahead of time to Eurordis for inclusion in the European Rare Disease Day central website**
- **Some ideas for possible activities:**
    - ❖ **Coordinate a letter writing or email campaign** to local or national policy and decision makers and authorities, to urge them to act for people living with rare diseases. The topics covered in the letter need to be adapted to the particular situation of rare disease patients in your region or country
    - ❖ **Send a press release to the media in your region / country** (see tools in section 13 for a press release template)
    - ❖ **Organise interviews with the media** to talk about the situation of rare disease patients in your region / country (magazines and newspapers, television, radio)
    - ❖ **Organise events** around rare diseases and the topics at heart of patients in your country (conference, workshops and meeting, walks, demonstrations, sports events etc)
    - ❖ **Approach a special/famous person** and ask him/her to be the patron of the day in your country, or use the existing patron of your organisation

- **Give out awards** to people who are recognised for having acted effectively or outstandingly for the cause of rare disease patients

- **Hold a competition** centred on rare diseases: photo competition, art contest, essays etc

- **Set up discussion forums**

- **Set up a help line to respond to enquiries or promote your existing help line**

- **Display posters, images or other awareness raising displays.** Images and videos can inspire many to take action. This toolkit includes posters, a logo and other tools that can be used for campaigning and at events

- **Distribute stickers and flyers** to people on the street, in schools, hospitals, or universities

- **Make appointments to meet with your local and national authorities,** in particular those that play a central role in research and the diagnosis, treatment and care of rare disease patients

- **Organise fund-raising events** or a special fund-raising campaign:

    - Collect donations from people on the street
    - Collect donations from your website
    - Organise a fund-raising dinner with a special guest
    - Special mailings
    - Sell specific items
    - Approach corporate organisations for donations or long-term partnerships

13. Useful tools

Lilwen, France, Aniridia

a. Press release for National Alliances

29 February 2008: A rare Day for very special people

1st European Rare Disease Day

In red: *information to be completed by National Alliances*

Place, Date – [Your National Alliance] is happy to announce the celebration of the 1st European Rare Disease Day in [your region or country] on 29 February 2008. Rare diseases are chronic, progressive, degenerative, and often life-threatening with high levels of pain. There is no cure today for the 6 to 8,000 rare diseases, 75% of which affect children. People living with rare diseases face similar problems, such as delay in correct diagnosis, lack of quality information, lack of appropriate healthcare and inequities in access to treatment and care. Research is still too scarce.

After years of fight by rare disease patient organisations such as [Your National Alliance] and EURORDIS (Rare Diseases Europe), rare diseases are now a public health priority at the European Union level. In [your region or country], there are x people living with rare diseases. [Elaborate on situation of rare disease patients in your region or country, what their needs are, and what is urgent to do……………………………………………………………………………………………
……………………………………………………………………………………………
…………………………………………………………………………………………….]
The European Rare Disease Day, on 29 February 2008, is the opportunity to celebrate the diversity of the rare disease community in [your region or country] and in Europe.

[Contact person]

b. Logos and visual identity

European
Rare Disease Day
Logo (to be
developed)

Download at: www.eurordis.org/xxx

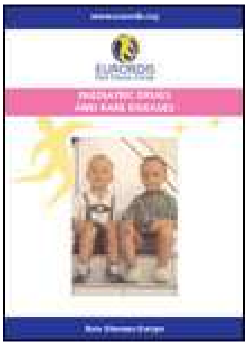
Member of
EURORDIS
Rare Diseases Europe

Download at: www.eurordis.org/yyy

Rare Disease Day
Poster (to be
developed)

Download at: www.eurordis.org/zzz

c. Fact sheets



i. What is a rare disease?
   http://www.eurordis.org/IMG/pdf/Fact_Sheet_RD.pdf

ii. What is an orphan drug?
   http://www.eurordis.org/IMG/pdf/Fact_Sheet_OD.pdf

iii. Rare disease patient groups in the EU
   http://www.eurordis.org/IMG/pdf/Fact_Sheet_PO.pdf

iv. Paediatric drugs and rare diseases
   http://www.eurordis.org/IMG/pdf/Fact_Sheet_Paediatrics.pdf

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